

554387

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property  
Organization  
International Bureau



(43) International Publication Date  
11 November 2004 (11.11.2004)

PCT

(10) International Publication Number  
WO 2004/096978 A2

- (51) International Patent Classification<sup>7</sup>: C12N
  - (21) International Application Number: PCT/IL2004/000181
  - (22) International Filing Date: 24 February 2004 (24.02.2004)
  - (25) Filing Language: English
  - (26) Publication Language: English
  - (30) Priority Data: 155588 27 April 2003 (27.04.2003) IL
  - (71) Applicant (for all designated States except US): METABOGAL LTD. [IL/IL]; P.O. Box 432, 11 013 Kiryat-Shmona (IL).
  - (72) Inventors; and
  - (75) Inventors/Applicants (for US only): SHAALTIEL, Yoseph [IL/IL]; Moshav Beit Hillel, 12 255 Upper Galilee (IL). BAUM, Gideon [IL/IL]; Kibbutz Ayelet HaShachar, 12200 Upper Galilee (IL). BARTFELD, Daniel [IL/IL]; 11 Havradim Street, 11631 Kiryat Shmona (IL).
  - (74) Agent: G. E. EHRLICH (1995) LTD.; 11 Menachem Begin Street, 52 521 Ramat-Gan (IL).
  - (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
  - (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).
- Published:**  
— without international search report and to be republished upon receipt of that report
- For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.*



WO 2004/096978 A2

(54) Title: PRODUCTION OF HIGH MANNOSE PROTEINS IN PLANT CULTURE

(57) Abstract: A device, system and method for producing glycosylated proteins in plant culture, particularly proteins having a high mannose glycosylation, while targeting such proteins with an ER signal and/or by-passing the Golgi. The invention further relates to vectors and methods for expression and production of enzymatically active high mannose lysosomal enzymes using transgenic plant root, particularly carrot cells. More particularly, the invention relates to host cells, particularly transgenic suspended carrot cells, vectors and methods for high yield expression and production of biologically active high mannose Glucocerebrosidase (GCD). The invention further provides for compositions and methods for the treatment of lysosomal storage diseases.